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Congress of the United States
House of Representatives
Subcommittee on Health and the Environment
of the
Committee on Energy and Commerce
Washington, D.C. 20515

TO BOB BERGER

FROM TIM WESTMORELAND (202) 225-4952

SEPTEMBER 23, 1982

ATTACHED IS A PIECE BY CONGRESSMAN WAXMAN ON ORPHAN DRUGS. THE LEGISLATION IS TO COME UP ON THE FLOOR OF THE HOUSE TUESDAY OF NEXT WEEK. WE HOPE IT WILL BE CONSIDERED IN THE SENATE SHORTLY THEREAFTER.

BETTY CUNIBERTI IS WORKING ON A PIECE FOR THE VIEW SECTION.

EVERYONE KNOWS ABOUT CANCER AND HEART DISEASE: THESE ILLNESSES ARE OFTEN FATAL AND, ALTHOUGH SOME TREATMENTS ARE KNOWN, MANY CASES ARE INCURABLE. THESE DISEASES ARE HUMAN AND SOCIAL TRAGEDIES. LARGE RESEARCH PROGRAMS--BOTH PUBLIC AND PRIVATE--HAVE BEEN UNDERWAY FOR A LONG TIME.

BUT THERE IS ANOTHER GROUP OF TRAGIC ILLNESSES THAT NOT EVERYONE KNOWS ABOUT: THERE ARE MILLIONS OF PEOPLE IN THIS COUNTRY WHO SUFFER FROM RARE DISEASES, DISEASES THAT DO NOT ATTRACT THE UNIVERSAL EFFORT THAT CANCER DOES.

FOR MANY OF THESE CONDITIONS, EFFECTIVE TREATMENT IS NOT KNOWN. BUT FOR MANY OTHERS, TREATMENTS OR EVEN CURES ARE KNOWN BUT UNAVAILABLE BECAUSE OF THE COMMERCIAL WORKINGS OF THE PHARMACEUTICAL BUSINESS.

A YOUNG MAN FROM LOS ANGELES RECENTLY APPEARED BEFORE MY SUBCOMMITTEE IN CONGRESS. HE TESTIFIED ABOUT HIMSELF:

"RIGHT NOW I DON'T HAVE ANY MEDICATION TO TAKE BECAUSE THERE ISN'T ONE.... RIGHT NOW MY [SPASMS] AREN'T VERY BAD.... BUT THEY ARE GOING TO GET WORSE AND I AM GOING TO BE GRUNTING OBSCENITIES AND ITCHING AND TWITCHING AND THERE IS VERY LITTLE I CAN DO ABOUT IT, AND I WILL PROBABLY JUST STAY AT HOME FOR A WHILE UNLESS THERE IS SOME RESEARCH, SOMETHING GETS DONE."

THE WITNESS HAS TOURETTE'S SYNDROME, A RARE CONDITION AFFECTING 100,000 PEOPLE. THE DISEASE CAUSES MUSCLE SPASMS, TICS, AND INVOLUNTARY SPEECH AND CURSING.

THERE ARE THOUSANDS OF OTHER PEOPLE IN THIS COUNTRY WHO HAVE ALS (LOU GEHRIG'S DISEASE); THOUSANDS OF OTHERS SUFFER FROM CYSTIC FIBROSIS; THOUSANDS MORE FROM TAY SACHS OR HUNTINGTON'S. IN SOME CASES MORE RESEARCH NEEDS TO BE DONE TO FIND DRUGS THAT WILL HELP. THAT RESEARCH SHOULD BE ENCOURAGED.

BUT ALSO, TOO OFTEN, A DRUG THAT COULD HELP IS KNOWN BY RESEARCHERS, BUT PATIENTS CAN'T GET IT. WHY? SIMPLY BECAUSE THE MARKET FOR A DRUG FOR A RARE ILLNESS (A SO-CALLED "ORPHAN DRUG") IS NOT LARGE ENOUGH TO BRING A PROFIT.

SIMPLY PUT, NOT ENOUGH PEOPLE ARE SICK. THE PHARMACEUTICAL COMPANY KNOWS IT WON'T MAKE MONEY. THE SITUATION IS A TRAGEDY THAT FRUSTRATES AND ANGERS VICTIMS, THEIR FAMILIES, AND THEIR PHYSICIANS.

A DOCTOR DOING RESEARCH ON ONE ORPHAN DRUG REPORTED TO THE HOUSE SUBCOMMITTEE ON HEALTH AND THE ENVIRONMENT, WHICH I CHAIR, ABOUT THE PROBLEMS HE HAS HAD. FOR SEVEN YEARS HE HAS BEEN WORKING WITH A DRUG FOR A KIDNEY DISEASE THAT OCCURS IN ABOUT 100 CHILDREN A YEAR. THE DISEASE--CYSTINOSIS--IS ALWAYS FATAL, USUALLY BY AGE TEN.

HIS DRUG CAN EFFECTIVELY CURE IT, BUT HE CAN'T GET IT ON THE MARKET.

TODAY FOR THESE CHILDREN THE ONLY ALTERNATIVE IS KIDNEY TRANSPLANTS, WHICH COST FEDERAL TAXPAYERS ABOUT $3 MILLION A YEAR. BRINGING THIS DRUG TO MARKET WOULD COST ABOUT ONE PERCENT OF THAT.

OUR PRIVATE DRUG DEVELOPMENT SYSTEM, WHICH HAS GIVEN US SO MANY OTHER PROMISING DRUGS, HAS A CRACK THROUGH WHICH PEOPLE WITH RARE DISEASES FALL. THERE ARE NO INCENTIVES FOR HUGE PHARMACEUTICAL COMPANIES TO PRODUCE ORPHAN DRUGS.

BUT THE VICTIMS OF THESE DISEASES DO NOT SUFFER LESS BECAUSE THEY ARE SO FEW. IN FACT THE KNOWLEDGE THAT THEIR TREATMENT IS UNAVAILABLE JUST BECAUSE IT DOESN'T TURN A PROFIT ADDS CORPORATE INSULT TO THEIR ILLNESS.

NO ONE QUESTIONS THE RIGHT OF THE PHARMACEUTICAL INDUSTRY TO MAKE A RETURN ON ITS INVESTMENTS. BUT THOSE WHO PROFIT FROM THE ILLNESS OF OTHERS ALSO HAVE PUBLIC RESPONSIBILITY TO DO SOMETHING MORE THAN DEVELOP ANOTHER SLEEPING PILL OR TRANQUILIZER.

SOMETHING MUST BE DONE TO CORRECT THIS SITUATION. IT IS AN UNACCEPTABLE STATE OF AFFAIRS WHEN PEOPLE ARE ALLOWED TO SUFFER BECAUSE THEIR ILLNESS DOESN'T MAKE MONEY.

IN HEARINGS BEFORE MY SUBCOMMITTEE, MEMBERS OF THE PHARMACEUTICAL INDUSTRY HAVE DESCRIBED BARRIERS TO THE DEVELOPMENT OF OPRHAN DRUGS. I HAVE INTRODUCED A BILL THAT EASES REGULATORY BARRIERS AND PROVIDES FINANCIAL INCENTIVES TO THE INDUSTRY. THE BILL NOW HAS 171 CO-SPONSORS AND WILL BE VOTED ON IN THE HOUSE OF REPRESENTATIVES ON SEPTEMBER 28.

DURING THE LAST TWO YEARS, THE SUBCOMMITTEE HAS LEARNED A GREAT DEAL ABOUT ORPHAN DRUGS AND THOSE WHO SUFFER FROM RARE DISEASES. PHARMACEUTICAL COMPANIES HAVE TOLD US OF DIFFICULTY WITH THE DEVELOPMENT OF DRUGS. REPRESENTATIVES OF VARIOUS DISEASE ORGANIZATIONS HAVE TOLD US OF THEIR PLIGHT AND ASKED FOR OUR HELP. GOVERNMENT REGULATORS HAVE RAISED CONCERNS ABOUT THE SITUATION AND POSSIBLE SOLUTIONS.

THERE ARE THREE MAJOR PROBLEMS THAT HAVE BEEN DISCUSSED: MANY ORPHAN DRUGS CANNOT BE PATENTED AND MOST ARE NOT PROFITABLE. THE ANIMAL AND HUMAN TESTING REQUIRED IS COSTLY. AND THE DRUG APPROVAL PROCESS IS TOO UNCERTAIN.

THE ORPHAN DRUG ACT RESPONDS TO THESE PROBLEMS.

BECAUSE OF FEDERAL PATENTS, NEW PHARMACEUTICALS ARE PHENOMENALLY PROFITABLE. SINCE SOME ORPHAN DRUGS ARE ARE NOT PATENTABLE AND ALL ARE--BY DEFINITION--FOR A SMALL GROUP OF CONSUMERS, THEY CANNOT RETURN SIMILAR PROFITS. OUR BILL CREATES A SEVEN-YEAR EXCLUSIVE MARKETING RIGHT FOR THOSE ORPHAN DRUGS THAT CANNOT BE PATENTED. AS THE SOLE

SUPPLIER OF A TREATMENT, A COMPANY WOULD BE ABLE TO RECOUP AT LEAST PART OF ITS INVESTMENT. THE BILL ALSO PROVIDES A TAX CREDIT FOR THE AMOUNT SPENT ON HUMAN TESTING TO REDUCE THE OVERALL COST OF DEVELOPMENT AND MARKETING.

IN ADDITION, THE BILL STREAMLINES THE DRUG APPROVAL PROCESS. COMPANIES WILL KNOW SOONER WHAT THE FOOD AND DRUG ADMINISTRATION EXPECTS THEM TO DO.

WE HAVE ALSO ANSWERED THE CONCERNS OF VARIOUS CONSUMER GROUPS AND DISEASE ORGANIZATIONS: WE HAVE ALLOWED GREATER ACCESS TO ORPHAN DRUGS THAT ARE UNDER INVESTIGATION. THE BILL ALSO ESTABLISHES A COMMISSION ON ORPHAN DRUG DEVELOPMENT, WHICH WOULD BE RESPONSIBLE FOR COORDINATING DRUG DEVELOPMENT EFFORTS BY FEDERAL AGENCIES AND PROMOTING THE DEVELOPMENT AND MARKETING OF ORPHAN DRUGS BY THE PRIVATE SECTOR.

SOME OF THE PHARMACEUTICAL MANUFACTURERS HAVE ARGUED THAT ORPHAN DRUGS SHOULD NOT BE TREATED DIFFERENTLY FROM OTHER DRUGS. THEY SAY WE SHOULD GIVE THEM REGULATORY RELAXATION FOR THE APPROVAL OF ALL DRUGS.

BUT ORPHAN DRUGS <u>ARE</u> DIFFERENT. ONLY 34 HAVE BEEN DEVELOPED AND MARKETED IN THE LAST TEN YEARS, AND OF THESE, 24 WERE FULLY OR PARTIALLY FUNDED BY THE FEDERAL GOVERNMENT OR THROUGH UNIVERSITY COLLABORATION. THESE STATISTICS ARE AN UNHAPPY COMMENTARY ON THE INDUSTRY.

RECENTLY, THE SUBCOMMITTEE CONDUCTED A SURVEY TO COMPILE INFORMATION ON ALL KNOWN ORPHAN DRUGS. WE POLLED PHARMACEUTICAL COMPANIES, UNIVERSITY RESEARCHERS, AND FEDERAL AGENCIES. I SUSPECT THAT WE NOW HAVE THE MOST COMPREHENSIVE MATERIAL AVAILABLE ON THE SUBJECT.

THE SUBCOMMITTEE'S SURVEY CONFIRMED SOME STATEMENTS WE HAD HEARD DURING HEARINGS.

*ORPHAN DRUGS ARE NOT PROFITABLE.

*IT IS DIFFICULT TO CONDUCT HUMAN TRIALS OF ORPHAN DRUGS BECAUSE THERE ARE SO FEW PEOPLE WITH ANY GIVEN DISEASE.

*MANUFACTURERS OF SOME OF THE DRUGS UNDER INVESTIGATION HAD NO INTENTION OF SEEKING APPROVAL TO MARKET THE DRUG.

*THE SURVEY FOUND A TOTAL OF 134 DRUGS FOR RARE DISEASES. ONLY 71 ARE EITHER MARKETED OR IN THE "INVESTIGATIONAL NEW DRUG" ("IND") STATUS, A REGULATORY STATE ALLOWING LIMITED AVAILABILITY.

THE SUBCOMMITTEE'S HEARINGS AND SURVEY HAVE MADE THE NEED FOR THIS LEGISLATION CLEAR. THE PROFIT SYSTEM FOR DRUGS HAS FAILED US. WE CANNOT CONTINUE TO ACCEPT THE CALCULUS THAT SMALL NUMBERS OF PATIENTS AND SMALL PROFITS ARE ROUNDED DOWN TO NO RESEARCH AND NO TREATMENT.

THE ORPHAN DRUG ACT WILL NOT GUARANTEE THAT DRUGS FOR RARE DISEASES WILL BE DEVELOPED. IT WILL, HOWEVER, EASE BARRIERS AND PROVIDE FINANCIAL INCENTIVES. WITH THESE CHANGES, IF THE PHARMACEUTICAL INDUSTRY DOES NOT MEET THE CHALLENGE OF DEVELOPING AND MARKETING MORE ORPHAN DRUGS, THEY WILL BE DOING THE AMERICAN PEOPLE A GRAVE DISSERVICE THAT NEITHER THE CONGRESS NOR THE PUBLIC CAN ACCEPT.